



# ERICA

European Rare Disease Research  
Coordination and Support Action



# The value of WP25.4 TASK for ERNs in ERDERA



Maurizio Scarpa –ALEXIS ARZIMANOGLOU

MetabERN-EPICARE

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## RATIONAL OF TASK 25.4

The whole chain of clinical research remains a major challenge to cover the unmet needs within the RD community and will need the support of the ERN capacity (with hundreds HC providers) to address the patient needs, not only in terms of drug development but also considering device development. ERNs are specialized networks that connect healthcare providers across Europe to improve diagnosis, treatment, and care for patients with rare diseases.

Their contributions to the ERDERA project include:

- **Facilitating Knowledge Sharing:** ERNs enable the exchange of expertise and best practices among healthcare professionals, researchers, and institutions involved in rare disease management.
- **Enhancing Research Collaboration:** By linking various stakeholders, ERNs foster collaborative research efforts that can lead to innovative treatments and diagnostic methods tailored to rare diseases.
- **Patient-Centric Approaches:** ERNs emphasize the importance of patient involvement in research and treatment processes, ensuring that the needs and perspectives of patients are prioritized.

## FACILITATING COLLABORATION AND NETWORKING

- **Connecting Experts:** Action: Task 25.4 does serve as the ERN platform that link healthcare professionals, researchers, and institutions specializing in rare diseases. This networking is essential for sharing knowledge, expertise, and best practices.

-**To align and integrate the strategic research roadmap of the ERNs in the RDP and beyond:** Action: Create a complete search of research projects running in the ERNs in order to understand the level of engagement of the single ERN and potentially identify common field of research to be funded in specific projects (crossing diseases, pathophysiology projects, etc.)

-**Public-Private Partnerships:** Action: Task 25.4 will play a role in fostering collaborations between public institutions and private entities, enhancing research and development efforts in the field of rare diseases.

### Enhancing Research and Innovation

- Driving Research Initiatives: ERNs contribute to the design and implementation of research projects aimed at understanding rare diseases better and developing innovative therapies.
- Clinical Trial Support: They facilitate access to clinical trial networks, ensuring that patients with rare diseases can participate in trials for new treatments, thus accelerating the development process.

## ERN LIVING LAB

The ERN Living Lab, is aimed to identify research priorities in the RD arena, creation of competitive research teams embracing basic and translational research, to facilitate participation in competitive grants and hence to enable breakthroughs in diagnosis and therapy progress. The ERN's Living Lab initiative is part of a broader effort to enhance research and innovation in the field of RDs.

This initiative aims to create a collaborative environment where stakeholders—including patients, researchers, healthcare providers, and industry partners—can work together to develop and test new solutions for the diagnosis and treatment of rare diseases.

## ACTIONS

The deployment of the Living Lab initiative will involve several key activities:

- **Establishment of Collaborative Networks:** Building strong connections between various stakeholders, including industry, regulatory bodies, and patient organizations, to foster collaboration and resource sharing
- **Data Sharing and Integration:** Creating frameworks for sharing high-quality data across different platforms to enhance research capabilities and facilitate clinical trials
- **Training Programs:** Developing comprehensive training programs aimed at equipping researchers and healthcare professionals with state-of-the-art competencies in rare disease research
- **Patient Engagement Frameworks:** Implementing structured frameworks for patient involvement in research processes, ensuring their perspectives are integrated into all stages of research and innovation

The 25.4 task will ensure that, inside the ERN Living Lab, the ERN-CG represent a dissemination point for the benefit of ERN HCPs members about the activities performed inside the RDP, and, vice versa, it will provide the RDP with advices on the research priorities distilled from thematic workshops aimed at the identification of potential solutions to patients' unmet needs.

A constant collaboration will be ensured with other Programs such as IRDIRC and EU projects such as “Realised” on new clinical trials strategies and the Joint Action JARDIN, for which this task will contribute by proposing the ERN-CG as advisory body in order to facilitate that ERNs have a common voice in the JARDIN implementing actions

## HOW TO IMPLEMENT THE ERN RESEARCH STRATEGY IN ERDERA

### 1. Develop a Governance Structure:

- Establish governance frameworks that define roles and responsibilities among various stakeholders, including researchers, healthcare providers, and patient organizations.
- Create decision-making bodies or committees that can prioritize research initiatives and harmonize efforts across different ERNs.

### 2. Facilitate Collaboration:

- Promote networking and information-sharing platforms that encourage ERNs to share knowledge, data, and best practices.
- Organize joint workshops, conferences, and training sessions to foster collaboration between ERNs and enhance research capabilities.

### 3. Create a Central Repository:

- Develop a database or platform where research data related to rare diseases can be stored and accessed by all ERNs. This would support data sharing and collaboration on large-scale research projects.
- Ensure compliance with ethical standards and regulations for data management, particularly regarding patient privacy.

### 4. Funding and Resource Allocation:

- Identify and secure funding sources, including EU grants and partnerships with private industries, to support collaborative research initiatives.
- Allocate resources efficiently to address the highest-priority research areas identified in collaboration with ERNs.

### 5. Engage Stakeholders:

- Involve patients, caregivers, and advocacy groups in the research process to ensure that research priorities reflect the needs and experiences of those affected by rare diseases.
- Create advisory panels that include stakeholders from different backgrounds to guide research directions and priorities.

## COLLABORATION BETWEEN ERNS

### 1. Shared Research Platforms:

- Establish joint research platforms to enable ERNs to embark on collective research projects targeting overlapping rare disease areas.
- Engage in cross-network collaborations that facilitate multi-disciplinary approaches to complex research questions.

### 2. Standardized Protocols and Methodologies:

- Develop standardized methodologies and protocols for conducting research across different ERNs, ensuring consistency and reliability of results.
- Encourage sharing of best practices related to clinical trials, data collection, and analysis.

### 3. Joint Funding Applications:

- Foster the development of consortiums to apply for joint funding opportunities, thereby increasing chances of securing financial support for research initiatives.



## RESEARCH PRIORITIES FOR ERNs

### 1. Identification of Gaps in Knowledge:

- Conduct comprehensive assessments to identify gaps in current knowledge related to rare diseases that require further investigation, including epidemiology, treatment outcomes, and patient experiences.

### 2. Focus on Novel Therapies and Treatments:

- Prioritize research on innovative therapies, including gene therapy, personalized medicine, and repurposing existing drugs for rare diseases.

### 3. Development of Diagnostic Tools:

- Encourage research initiatives aimed at developing better diagnostic tools and methodologies to facilitate early and accurate diagnosis of rare diseases.

### 4. Patient-Centered Research:

- Promote research that prioritizes patient outcomes and experiences, ensuring that studies address the challenges faced by patients with rare diseases.

### 5. Health Economics and Access:

- Investigate the health economic aspects of rare diseases to understand the costs associated with different treatments, and work towards improving access to effective therapies.

## CREATING AN IDENTITY OF RESEARCH FOR ERNs

### 1. Branding and Dissemination:

- Develop a coherent branding strategy that highlights the unique contributions of ERNs to rare disease research, working to build awareness and recognition within the research community and among the general public.

### 2. Publication and Advocacy:

- Encourage research findings to be published in reputable journals and presented at conferences, effectively disseminating knowledge and showcasing the importance of ERNs in advancing rare disease research.

### 3. Continuous Evaluation and Adaptation:

- Implement mechanisms for continuous feedback and evaluation of research initiatives, allowing ERDERA to adapt and refine its strategies based on changing scientific landscapes and emerging needs in the field of rare diseases.

## ROADMAP FOR IMPLEMENTING THE ERNs RESEARCH STRATEGY

- **Establishing a Comprehensive Ecosystem:** ERDERA aims to create a robust ecosystem that integrates funding, clinical research networks, and supporting services. This structure will enhance the readiness for clinical trials and accelerate the translation of research into improved patient care.
- **Public-Private Partnerships:** Strengthening collaborations between public and private sectors is essential. This includes fostering relationships that can drive innovation across the entire research and development (R&D) value chain.
- **Patient-Centric Research:** The alliance will focus on research that is led by patient needs. This approach ensures that the outcomes are relevant and beneficial to those affected by rare diseases.
- **Digital Transformation:** Leveraging digital tools to connect patient data with research activities will be critical. This integration will facilitate better diagnostics, treatment pathways, and overall healthcare delivery for rare disease patients.

## RESEARCH PRIORITIES FOR ADVANCING RARE DISEASES

To advance progress in rare diseases, WP24.5 will identify specific research priorities within the ERNs:

- **Translational Research:** Focus on translating basic research findings into clinical applications. This includes developing new therapies and diagnostic methods tailored for rare diseases.
- **Patient Empowerment:** Research should also prioritize educational initiatives that empower patients and their families, enabling them to participate actively in their care and treatment decisions.
- **Regulatory Compliance:** Ensuring that all research activities comply with existing regulations while advocating for policies that facilitate innovation in rare disease treatments.
- **Collaboration with Regulatory Bodies and stakeholders:** Engaging with regulatory agencies and stakeholders to streamline the development and approval processes for new therapies developed through collaborative research efforts.

## STRATEGIES TO ADDRESS FUNDING FRAGMENTATION

- **Integrated Funding Mechanisms:** ERDERA plans to establish a comprehensive funding structure that consolidates various funding sources, including public, private, and philanthropic contributions. This integrated approach will help streamline financial support for research initiatives across ERNs.
- **Public-Private Partnerships:** By fostering collaborations between public institutions and private entities, ERDERA can leverage additional resources and expertise. These partnerships are designed to create a more sustainable funding environment for rare disease research.
- **Targeted Funding Calls:** ERDERA will issue specific calls for funding that align with the strategic research priorities identified within the ERNs. This targeted approach ensures that resources are allocated efficiently to areas of greatest need and potential impact.
- **Capacity Building:** ERDERA will focus on enhancing the capacity of ERNs to attract funding by providing training and support in grant writing, project management, and collaboration techniques. This capacity-building initiative aims to empower networks to compete more effectively for available funds.
- **Coordination and Networking:** Establishing a strong coordination framework among ERNs will facilitate better communication and collaboration in identifying joint funding opportunities. By working together, ERNs can present a united front to funders, increasing their chances of securing financial support.

## COORDINATION MECHANISMS

- **Establishment of a Centralized Framework:** WP24.5 will create a centralized framework that serves as a hub for collaboration among various stakeholders, including national health authorities, research institutions, and patient organizations. This framework will facilitate the sharing of best practices and research priorities across regions.
- **Regular Stakeholder Meetings:** By organizing regular meetings and workshops, ERDERA will promote dialogue among national and regional entities. This will help align their research agendas with the overarching goals of the ERNs and ensure that local strategies are compatible with European objectives.
- **Development of Joint Research Agendas:** ERDERA will work with ERNs to develop joint research agendas that reflect both national priorities and European needs. This collaborative approach ensures that research efforts are not duplicated and resources are utilized efficiently.

## CAPACITY BUILDING

- **Training Programs:** ERDERA will provide training programs aimed at enhancing the skills of researchers and healthcare professionals in the field of rare diseases. These programs will focus on best practices in research collaboration, grant writing, and project management.
- **Support for Policy Development:** ERDERA will assist national governments in developing policies that support rare disease research. This includes providing evidence-based recommendations that align with European strategies.

By implementing these strategies, ERDERA aims to create a cohesive and integrated approach to rare disease research across Europe, ensuring that national and regional strategies are effectively coordinated and aligned with broader European objectives.